# Morphological and functional integration of stem cell derived retina organoid sheets into degenerating retina models

## Grant Award Details

Morphological and functional integration of stem cell derived retina organoid sheets into degenerating retina models

**Grant Type:** Therapeutic Translational Research Projects

**Grant Number:** TRAN1-10995

**Investigator:**

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| **Name:** | Magdalene Seiler |
| **Institution:** | University of California, Irvine |
| **Type:** | PI |

**Disease Focus:** Retinitis Pigmentosa, Vision Loss

**Human Stem Cell Use:** Embryonic Stem Cell

**Cell Line Generation:** Embryonic Stem Cell

**Award Value:** $4,769,039

**Status:** Pre-Active

## Grant Application Details

**Application Title:** Morphological and functional integration of stem cell derived retina organoid sheets into degenerating retina models

**Public Abstract:**

**Translational Candidate**

Retina organoid sheets (ROs) derived from CSC14 human embryonic stem cells (NIH registry line #0284) manufactured under GMP conditions

**Area of Impact**

Retinitis PIgmentosa (RP) (irreversible loss of photoreceptors) due to mutation of photoreceptors and/or other retinal genes

**Mechanism of Action**

Proposed mechanism of action is cell replacement, combined with trophic effects. Transplanted hESC-derived retina organoid sheets will mature into photoreceptors and integrate with the degenerate recipient's retina. Such transplants have improved visual acuity and responses to flashes of light in the midbrain (superior colliculus) of immunodeficient retinal degenerate rats (two different models).

**Unmet Medical Need**

There is currently no treatment for retinitis pigmentosa which is designated an Orphan disease by the FDA. Therapies in current clinical trials only target trophic effects which are only effective in early stages to delay degeneration.

**Project Objective**

Pre-IND meeting

**Major Proposed Activities**

- Establishment of Working Cell Bank, GMP implementation of retina organoid (RO) production, establish product specification and release criteria
- Identify and demonstrate markers correlated with function after maturation in vitro; functional in vitro imaging (FLIM and HSpec)
- In vivo pharmacology: demonstrate efficacy in immunodeficient and -competent rat model and in immunocompetent rabbit model of RP,

**Statement of Benefit to California:**

Retinal diseases reduce the quality of life of patients, at significant cost to the health care system. The proposed replacement therapy is the only one that targets more mature disease stages of RP, for which no other therapy exists. An effective treatment will keep afflicted individuals productive, enhance State tax revenues and defray the healthcare cost burden to taxpayers. It will also lead to robust industry developments, effectively leading to job creation and tax benefits.

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